United States Senate
SPECIAL COMMITTEE ON AGING
WASHINGTON, DC 20510–6400
(202) 224–5364

November 4, 2025

The Honorable Martin Makary, MD
Commissioner
Food and Drug Administration
10903 New Hampshire Avenue
Silver Spring, MD 20993

Dear Commissioner Makary:

We write to express the U.S. Senate Special Committee on Aging's interest in the Food and Drug Administration's (FDA) ongoing efforts to support innovation in the development and approval of treatments for rare diseases. Over 30 million Americans are living with at least one of the more than 7,000 rare diseases, with about 90% having no FDA-approved treatment.[1] While we use the term "rare disease," the reality is that roughly one in 10 Americans suffer from a rare disease, making them even rarer. The FDA's leadership and scientific expertise must work quickly and effectively to ensure that hope becomes reality for these patients and their families.

As you know, Congress has provided the FDA with essential tools and flexibility to encourage innovation for rare disease therapies, including through the *21st Century Cures Act* (P.L. 114-255) and the *Accelerating Access to Critical Therapies Act* (P.L. 117-79). We are encouraged by the agency's recent initiatives to establish a Rare Disease Innovation Hub and by the release of the Rare Disease Evidence Principles from the Center for Drug Evaluation and Research and the Center for Biologics Evaluation and Research to help modernize the process.

To address the issues faced by many Americans, including our nation's seniors, the U.S. Senate Special Committee on Aging will hold a hearing focusing on the rare disease drug development pipeline and the unique regulatory challenges that can affect patients' access to potentially lifesaving therapies. Our goal is to better understand how the FDA supports innovation while upholding the highest standards of safety and scientific integrity. We are eager to learn about the progress made this year, identify opportunities for continued improvement, and reinforce our shared commitment to assisting patients who often have no other options.

We would like to know more about the various initiatives undertaken by the

[1] https://rarediseases.org/understanding-rare-disease/rare-disease-facts-and-statistics/

FDA, under your leadership, to address the unique challenges facing the rare disease community, and how to increase the speed and number of rare disease drug approvals.

We appreciate the FDA's leadership in ensuring that the United States remains the world's leader in biomedical innovation. We look forward to continuing to work with you and your team as we prepare for this important discussion.

Sincerely,

Rick Scott
Chairman
Senate Special Committee on Aging

Kirsten E. Gillibrand
Ranking Member
Senate Special Committee on Aging